Q&A

The "Patient Journey" to Real-World Evidence: An Interview With Tanisha Carino

Value & Outcomes Spotlight's had the chance to sit down with Tanisha Carino, PhD, a respected senior executive with more than 2 decades of experience in academia, government, and the private sector, to gain a further understanding on the 21st Century Cures Act. She joined the Milken Institute in January 2018 as executive director of *FasterCures*, the center devoted to saving lives and improving the medical research system. Throughout her distinguished career, including GlaxoSmithKline, Avalere Health, and Medicare, Carino has been at the forefront of collaborative efforts to promote policies, research, and business practices that support the fight against disease and improve the lives of patients. Carino earned her PhD in health policy from Johns Hopkins University.

FasterCures, a Center of the Milken Institute, has a singular goal: to save lives by speeding science to all patients. For over 15 years, *FasterCures* has created a global community dedicated to accelerating medical solutions and has worked with leaders across biomedical research to accelerate medical research, including the passage of 21st Century Cures.

***Value & Outcomes Spotlight:* How do you feel about our progress over the past 4 to 5 years in incorporating the patient voice into real-world evidence?**

Tanisha Carino: While the promise of "big data" has existed for some time, figuring out how to harness it to impact patient outcomes has come of age with the advent of new tools, data sources, and ever-increasing patient engagement and stakeholder collaboration.

The rise of social media and advancements in digital health have connected and empowered patients to a level not seen in the past. This has led to an explosion of patient-generated data from an ever-expanding number of sources that offer not only new (sometimes continuous) data streams, but additional opportunities to engage patients and understand their perspectives, goals, and needs.

For example, this month we see the US Food and Drug Administration (FDA) turning to its Patient Engagement Advisory Committee for suggestions from patients on how the agency can leverage patient-driven platforms to better engage patients and collect real-world data. Digital health has brought new stakeholders to the table and is revolutionizing the way that data are collected, shared, and used, as well as changing patient care.

We are seeing increased collaboration to address the myriad challenges and uncertainties regarding how to best collect and utilize data for each intended use. Strides are being made in integrating data sources, developing standards, and identifying how to engage patients meaningfully and collect patient-generated data to help make medical product development more efficient and less burdensome for patients and to better align products with patient needs.

What are your thoughts on incorporation of the patient voice in clinical research design and real-world data collection—are things progressing there, too?

It is an exciting time in real-world data and evidence development, particularly as it relates to including patient perspectives in the

decision-making process of medical product development and delivery. We have the opportunity, with the scale-up of electronic health records and other types of digitally collected data, to have a more complete picture of patients' experiences of their disease or condition, as well as their treatments and care.

Practically speaking, we continue to struggle with some difficult issues—poor data quality and lack of data standardization make it challenging to derive meaningful insights, siloes remain among different types of data that could be relevant to patients, like their wearable data. Additionally, the differences between patient data generated in the controlled process of a clinical trial and data generated in the real-world context are challenging to interpret (statistical significance versus clinical meaningfulness). However, progress has been made toward tackling challenges related to data aggregation, through the PCORI-funded Clinical Data Research Networks and standardization of outcomes that matter to patients through the development of core outcome sets.

> Strides are being made in integrating data sources, developing standards, and identifying how to engage patients meaningfully and collect patient-generated data to help make medical product development more efficient and less burdensome for patients and to better align products with patient needs.

One critically important next step to ensure that momentum continues—and we can realize the promise of turning patient journeys into data—is to directly engage with patients and caregivers. Patient engagement requires a certain level of know-how in terms of patients understanding what comprises their health data, how they are being used to improve their care and drive research, and how they can be actively engaged in contributing their data, and their time, as part of their participation in research. Efforts are underway to support patient communities, including our Health Data Basics project as well as a newly created effort in the United Kingdom called Understanding Patient Data.

When you consider what is possible relative to that which is practical, how do you see "Cures" supporting better evidence development by incorporating the patient voice into real-world data?

We are currently seeing how 21st Century Cures has raised the importance of partnering with patients and how it will ultimately raise the profile of those organizations investing in early pilot projects to incorporate the patient voice in evidence generation using real-world data. Through requirements mandated by Cures, including issuing guidance on Patient-Focused Drug Development, the FDA is setting expectations for industry. In addition, as the patient experience section in the medical product label becomes populated with data, it will be interesting to see how that information is communicated to patients and providers, as well as used by decision makers in health care.

At the same time, patient organizations continue to function as critical partners in evidence development, from their support of basic and translational research and funding of natural history studies, to the establishment of patient registries and master protocols. However, these efforts require a high level of resources, and a need exists to support capacity building of these organizations, to engage more broadly and, for some, to collect and generate patient data.

What do you see as the next steps for the patient perspective propelling a data-driven journey moving forward?

The next steps exist at several levels and will require engagement from individuals, communities, and organizations.

First, patient groups play a critical role in both advocating for the unmet needs in their patient communities and catalyzing research and partners in data collection and decision making.

As patient groups play a bigger role across the ecosystem, they are determining their assets and resource needs, so being focused and strategic is critical. If we are truly going to leverage big data to accelerate research, we must include individual patients, caregivers, and patient communities in the generation and interpretation of the data to ensure that the evidence generated is important to them and serves their unmet needs.

This will take scaling up patient engagement efforts through the redesign of clinical trials to allow for virtual participation, integrating wearable and other types of data generated by patients, and building the knowledge and empowerment of patients to be more active participants with their health data.

Second, as part of their efforts to drive innovation in medical research, patient organizations can take greater latitude with the types of data they collect, data that traditional clinical research and outcomes research community view as "messy"—sleep, diet, activity level, and even shopping patterns. However, health and how we measure it require not only traditional clinical outcomes of interest, but also data on the true lived experience. More needs to be done to create a stronger bridge between research and patient communities that support collaboration, shared language, and advancing analytical methods.

Finally, decision making is in flux because the data used to make decisions are evolving, so gathering stakeholders from across the ecosystem to define what data have value for what types of decisions is a critical next step.